FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Peripheral and Central Nervous System Drugs Advisory Committee (PCNS) Meeting
FDA White Oak Campus, Building 31 Conference Center, the Great Room (Rm. 1503)
10903 New Hampshire Avenue, Silver Spring, Maryland
November 24, 2015

DRAFT AGENDA

The committee will discuss new drug application (NDA) 206031, drisapersen solution for injection, sponsored by BioMarin Pharmaceutical Inc., for the treatment of patients with Duchenne muscular dystrophy with mutations in the dystrophin gene that are amenable to treatment with exon 51 skipping as determined by genetic testing.

8:00 a.m.	Call to Order and Introduction of Committee	**G. Caleb Alexander, MD, MS** Chairperson, PCNS
8:05 a.m.	Conflict of Interest Statement	**Phil Bautista, PharmD** Acting Designated Federal Officer, PCNS
8:10 a.m.	FDA Introductory Remarks	**Billy Dunn, MD** Director, Division of Neurology Products (DNP) Office of Drug Evaluation I (ODE I) Office of New Drugs (OND), CDER, FDA
8:15 a.m.	**SPONSOR PRESENTATIONS**	**BioMarin Pharmaceutical Inc.**
	Introduction	**Camilla V. Simpson, MSc** Group Vice President Regulatory Affairs and Pharmacovigilance BioMarin Pharmaceutical Inc.
	Duchenne Muscular Dystrophy: Natural History and Clinical Trial Considerations	**Craig M. McDonald, MD** Professor and Chair Department of Physical Medicine & Rehabilitation Director, Neuromuscular Disease Clinics University of California, Davis
	Efficacy of Drisapersen	**Henry J. Fuchs, MD** Chief Medical Officer BioMarin Pharmaceutical Inc.
	Safety of Drisapersen and Risk Management	**Giles V. Campion, MD, PhD** Group Vice President, Clinical Science BioMarin Pharmaceutical Inc.
	Summary of Benefit-Risk Clinical Perspective	**Craig M. McDonald, MD**
	Conclusion	**Henry J. Fuchs, MD**

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DRAFT AGENDA (cont.)

9:30 a.m.	Clarifying Questions	
9:45 a.m.	**BREAK**	
10:00 a.m.	**FDA PRESENTATIONS**	
	FDA Efficacy Review	**Veneeta Tandon, PhD** Clinical Reviewer DNP, ODE I, OND, CDER, FDA **Ashutosh Rao, PhD** Acting Chief Laboratory of Applied Biochemistry Division of Biotechnology Review & Research III Office of Biotechnology Products Office of Pharmaceutical Quality, CDER, FDA **Sharon Yan, PhD** Mathematical Statistician Division of Biometrics I, Office of Biostatistics Office of Translational Sciences, CDER, FDA
	Drisapersen Safety	**Evelyn Mentari, MD, MS** Clinical Safety Reviewer DNP, ODE I, OND, CDER, FDA
11:15 a.m.	Clarifying Questions	
11:30 p.m.	**LUNCH**	
12:30 p.m.	Open Public Hearing	
2:30 p.m.	Questions to the Committee/Committee Discussion	
3:30 p.m.	**BREAK**	
3:45 p.m.	Questions to the Committee/Committee Discussion	
5:30 p.m.	**ADJOURNMENT**	